Reference number(s)
2796-A

SPECIALTY GUIDELINE MANAGEMENT

GAMIFANT (emapalumab-lzsg)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications
Gamifant is indicated for the treatment of adult and pediatric (newborn and older) patients with primary hemophagocytic lymphohistiocytosis (HLH) with refractory, recurrent or progressive disease or intolerance with conventional HLH therapy.

All other indications are considered experimental/investigational and not medically necessary.

II. REQUIRED DOCUMENTATION

The following information is necessary to initiate the prior authorization review: medical record documentation (i.e., chart notes or laboratory report) confirming the diagnosis of HLH with the presence of one of the following: A) a mutation in one of the following genes: PRF1, UNC13D, STX11 and STXBP2, or B) presence of at least 5 clinical signs and symptoms of disease. (See Appendix A)

III. CRITERIA FOR INITIAL APPROVAL

Primary HLH
Authorization of 6 months may be granted for treatment of primary HLH when all of the following criteria are met:
A. Member has refractory, recurrent or progressive disease or intolerance with conventional HLH therapy.
B. Member's diagnosis of primary HLH was confirmed by either of the following:
    1. Mutation in one of the following genes: PRF1, UNC13D, STX11 and STXBP2
    2. Presence of at least 5 clinical signs and symptoms of HLH (See Appendix A)
C. Possible causes of secondary or acquired forms of HLH (e.g., autoimmune disease, persistent infection, malignancy, or loss of inhibitory immune mechanisms) have been ruled out.
D. Member has been evaluated for tuberculosis (TB) risk factors and has undergone pretreatment screening for latent TB with the purified protein derivative (PPD) skin test or interferon gamma release assay.
E. If member has a positive test result or is at risk for TB, prophylactic treatment for TB must be initiated before starting therapy.

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for primary HLH who have achieved or maintained positive clinical response.

V. APPENDIX

CLINICAL SIGNS AND SYMPTOMS OF HEMOPHAGOCYTIC LYMPHOHISTIOCYTOSIS (HLH)

1. Fever
2. Splenomegaly
3. Cytopenias (affecting at least 2 of 3 lineages in the peripheral blood: hemoglobin less than 9 g/dL [hemoglobin less than 10 g/dL in infants younger than 4 weeks], platelets less than 100,000/microliter, neutrophils less than 1,000/microliter)
4. Hypertriglyceridemia (fasting triglyceride greater than or equal to 265 mg/dL) or hypofibrinogenemia (less than or equal to 150 mg/dL)
5. Hemophagocytosis in bone marrow or spleen or lymph nodes or liver with no evidence of malignancy
6. Low or absent natural killer (NK) cell activity
7. Ferritin greater than or equal to 500 ng/mL
8. Soluble CD25 (soluble IL-2 receptor alpha) level greater than or equal to 2400 U/mL, or above age-adjusted, laboratory-specific normal levels (defined as 2 standard deviation from the mean)

VI. REFERENCES

1. Gamifant [package insert]. Waltham, MA: Sobi, Inc.; May 2022.
2. Henter JI, Horne A, Arico M et al. HLH-2004: diagnostic and therapeutic guidelines for hemophagocytic lymphohistiocytosis. Pediatr Blood Cancer. 2007;48:124-131.
3. Allen CE and McClain KL. Hematology Am Soc Hematol Educ Program. 2015;2015:177-82.
4. Janka, G.E. and E.M. Schneider, Modern management of children with haemophagocytic lymphohistiocytosis. Br J Haematol, 2004. 124(1): p. 4-14.